CIRM Funded Clinical Trials

A Phase I Clinical Trial for a Lentiviral Gene Therapy Targeting the TCIRG1 Gene for Infantile Malignant Osteopetrosis (IMO)

Disease Area:	Bone or Cartilage Disease
Investigator:	Gayatri Rao
Institution:	Rocket Pharmaceuticals, Inc.
CIRM Grant:	CLIN2-12095
Award Value:	$3,728,485
Trial Sponsor:	Rocket Pharmaceuticals, Inc.
Trial Stage:	Phase 1
Trial Status:	Not yet recruiting
Targeted Enrollment:	2
ClinicalTrials.gov ID:	NCT04525352

Gayatri Rao

Details:
Rocket Pharmaceuticals is conducting a clinical trial using a gene therapy for infantile malignant osteopetrosis (IMO), a rare and life-threatening disorder that develops in infancy. IMO is caused by defective bone cell function, which results in blindness, deafness, bone marrow failure, and death very early in life.

The trial will use a gene therapy that targets IMO caused by mutations in the TCIRG1 gene. The team will take a young child's own blood stem cells and inserting a functional version of the TCIRG1 gene. The newly corrected blood stem cells are then introduced back into the child, with the hope of halting or preventing the progression of IMO in young children before much damage can occur.

Design:
Phase I Trial

Goal:
To evaluate safety and preliminary efficacy.